UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF MASSACHUSETTS

MARK A. CORBAN, Individually and on Behalf of All Others Similarly Situated, Plaintiff, v. SAREPTA THERAPEUTICS, INC., CHRIS GARABEDIAN, SANDY MAHATME, and ED KAYE, Defendants.	CIVIL ACTION NO. **JURY TRIAL DEMANDED**

CLASS ACTION COMPLAINT

Plaintiff Mark A. Corban, individually and on behalf of all other persons similarly situated, by their undersigned attorneys, for his complaint against Defendants, allege the following based upon personal knowledge as to himself and his own acts, and information and belief as to all other matters, based upon, *inter alia*, the investigation conducted by and through his attorneys, which included, among other things, a review of the Defendants' public documents, conference calls and announcements made by Defendants, United States Securities and Exchange Commission ("SEC") filings, wire and press releases published by and regarding Sarepta Therapeutics Inc., analysts' reports and advisories about the Company, and information readily obtainable on the Internet. Plaintiff believes that substantial evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery.

NATURE OF THE ACTION

1. This is a federal securities class action on behalf of a class consisting of all persons other than Defendants who purchased Sarepta Therapeutics Inc. ("Sarepta," "SRPT" or the "Company") securities during the period beginning July 24, 2013 through and including November 12, 2013 (the "Class Period"), seeking to recover damages caused by Defendants'

violations of the federal securities laws and to pursue remedies under §§ 10(b) and 20(a) of the Securities Exchange Act of 1934 (the "Exchange Act") and Securities Exchange Commission ("SEC") Rule 10b-5 promulgated thereunder against the Company and certain of its officers and directors ("Defendants").

JURISDICTION AND VENUE

2. The claims asserted herein arise under and pursuant to Sections 10(b) and 20(a) of the Exchange Act (15 U.S.C. §78j(b) and 78t(a)) and SEC Rule 10b-5 (17 C.F.R. §240.10b-5).

3. This Court has jurisdiction over the subject matter of this action pursuant to § 27 of the Exchange Act (15 U.S.C. §78aa) and 28 U.S.C. § 1331.

4. Venue is proper in this District pursuant to § 27 of the Exchange Act, 15 U.S.C. § 78aa and 28 U.S.C. § 1391(b), as Sarepta's principal place of business is located within this District and certain of the Individual Defendants reside within this District.

5. In connection with the acts, conduct and other wrongs alleged in this Complaint, Defendants, directly or indirectly, used the means and instrumentalities of interstate commerce, including but not limited to, the United States mail, interstate telephone communications and the facilities of the national securities exchange, NasdaqGS.

PARTIES

6. Plaintiff, Mark A. Corban, purchased Sarepta securities during the Class Period, as set forth in his Investor Certification attached hereto, at artificially inflated prices and has been damaged thereby.

7. Defendant Sarepta is an Oregon corporation with its principal executive offices located in Cambridge, Massachusetts. Sarepta describes itself as a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases.

8. Defendant Chris Garabedian ("Garabedian") was at all relevant times President, Chief Executive Officer and a director of Sarepta.

9. Defendant Sandy Mahatme ("Mahatme") was at all relevant times Senior Vice President and Chief Financial Officer of Sarepta.

10. Defendant Ed Kaye ("Kaye") was at all relevant times Senior Vice President and Chief Medical Officer of Sarepta. Kaye joined Sarepta in June 2011. Previously he served as Group Vice President for Clinical Development and Therapeutic Head for Lysosomal Storage Disorders and Neurodegenerative Diseases at Genzyme since 2007. Kaye held additional leadership roles in Clinical Development and Medical Affairs over 10 years at Genzyme and developed specific experience with pediatric neuromuscular conditions. He played a leadership role in gaining Myozyme's approval for Pompe Disease and oversaw collaborations in this field, including the development of ataluren for Duchenne Muscular Dystrophy (DMD). He received his medical education and pediatric training at Loyola University Stritch School of Medicine and University Hospital, child neurology training at Boston City Hospital, Boston University, and completed his training as a neurochemical research fellow at Bedford VA Hospital, Boston University.

SUBSTANTIVE ALLEGATIONS

Background

11. Sarepta describes itself in its SEC filings as a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. According to the Company, applying its proprietary, highly-differentiated and innovative platform technologies, it is able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action.

12. To date, the Company has not generated any material revenue from any product sales.

13. During the Class Period and presently the Company's primary focus was and is on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy drug candidates, including its lead product candidate, eteplirsen.

14. Sarepta's lead program focuses on the development of disease-modifying therapeutic candidates for Duchenne muscular dystrophy, or DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD.

15. Eteplirsen is Sarepta's lead therapeutic candidate for DMD. If it is successful in its development efforts, eteplirsen will address a severe unmet medical need.

16. The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to regulation by state authorities and the FDA in the United States and other regulatory authorities in other countries, with regulations differing from country to country. Marketing of Sarepta's product candidates, including eteplirsen, in the United States or foreign countries is not permitted until the required approvals are obtained from the FDA or other applicable foreign regulatory authorities.

17. In 2012, Sarepta completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, the Company initiated an open label extension study that it expected to be completed in late 2013 with the same participants from the original Phase IIb placebo controlled trial. According to the Company's Form 10-K annual report for the year ended 2012, it anticipated initiating a pivotal clinical trial for eteplirsen by the end of 2013 and commencing dosing in this trial in early 2014.

18. DMD is caused by a mutation in the dystrophin gene, which codes for the protein dystrophin, an important component of muscle tissue that is necessary for structural support, and eteplirsen works by targeting precursor RNA and forcing the cellular machinery to skip over exon 51 (the DNA segment that codes for the dystrophin mutation), resulting in an altered mRNA template. By creating an mRNA template that does not code for exon 51, the cells of patients diagnosed with DMD are thus able to produce functional dystrophin. To measure efficacy in DMD, Serapta utilizes the 6 minute walk test (6MWT). This clinical endpoint is exactly what it sounds like: how far can a patient walk in 6 minutes. The test is based on the belief that the ability of a patient with the disease, to walk will diminish over time until the patient becomes wheel chair bound. Thus, measuring the stabilization/lack of regression in walking ability *via* the 6MWT is clinically meaningful.

19. During the Class Period, Defendants made materially false or misleading statements concerning, among other things, (1) the prospects of the FDA's acceptance for consideration of a New Drug Application ("NDA") for eteplirsen based on its Phase IIb study data set, and (2) the significance of that data set.

Materially False and Misleading Statements Issued During the Class Period

20. On July 24, 2013, Sarepta issued a press release announcing its plans to submit a NDA in the second quarter of 2014 for approval of its lead product candidate eteplirsen:

> **Sarepta Therapeutics Announces Plans to Submit New Drug Application to FDA for Eteplirsen for the Treatment of Duchenne Muscular Dystrophy in First Half of 2014**
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> CAMBRIDGE, MA — (Marketwired) — 07/24/13 — Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced it plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the first half of 2014 for the approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). Eteplirsen is Sarepta's lead exon-skipping compound in development for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

> ***The decision to submit an NDA for eteplirsen in 2014 is based on productive interactions with the FDA in a meeting that occurred this week. That meeting was a follow-up to the FDA's review of two recently submitted summary documents that included data on dystrophin and clinical outcomes from the existing eteplirsen studies. The FDA stated in pre-meeting comments that the Agency is "open to considering an NDA based on these data for filing."*** The Agency, however, requested additional information related to the methodology and verification of dystrophin quantification. Sarepta believes the requests from the Agency can be addressed and incorporated into an NDA submission in the first half of 2014.
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> ***"We are encouraged by the feedback from the FDA and believe that data from our ongoing clinical study merits review by the Agency and will be sufficient for an NDA filing," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We plan to work closely with the FDA to prepare an NDA submission in the first half of 2014 as we continue to prepare for our confirmatory study and our manufacturing scale up."***
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> The Agency would not commit to declaring dystrophin an acceptable surrogate endpoint under the CFR 314 Subpart H Accelerated Approval pathway prior to an NDA filing and commented that a decision by the Agency to file "the NDA would not indicate that we have accepted dystrophin expression as a biomarker reasonably likely to predict clinical benefit. A filing would only indicate that the question merits review, and that we deem the data to be reviewable."
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> Sarepta anticipates submitting an NDA for eteplirsen in the first half of 2014; however, the exact timing of the submission will be dependent on further discussions and agreement with the FDA on the information needed for an acceptable filing. Sarepta also intends to have an End-of-Phase II meeting with the agency later this quarter to discuss the requirements for the Chemistry, Manufacturing, and Controls (CMC) section of the NDA.

(emphasis added).

21. On August 8, 2013, the Company held its earnings conference call for the second quarter ended June 30, 2013. Defendants Garabedian, Mahatme, and Kaye participated in and spoke during the call:

> [Garabedian:] A couple of weeks ago we announced the outcome of our follow-up meeting with the FDA that took place in July and announced our plan to submit an NDA or New Drug Application for eteplirsen in the first half of next year. ***This decision was based on discussions with the agency and the Division of Neurology Products where they indicated that based on the Phase II data we've shared with them, they are open to considering an NDA filing. This feedback is particularly encouraging because it recognizes that our Phase IIB study data***

set is sufficient for the FDA to consider a filing and allows us to initiate the first step of the registration process for approval of a new drug.

There are a number of distinct steps that form the registration process in getting a new therapy approved by the FDA. The first step is the submission of a New Drug Application by a sponsor which is essentially a request by the sponsor asking the FDA to consider approving a new drug for a particular disease. Often a sponsor does not seek guidance from the FDA on the feasibility of whether or not an NDA submission would be acceptable for filing and review because there are general guidelines on the evidence that is required to consider an NDA for filing. However because Sarepta studies with eteplirsen produced a compelling and favorable data set on a mid stage or smaller trial it was important for us to gain feedback from the FDA if an NDA filing would be acceptable.

Specifically, we wanted to understand if it was possible for the FDA to consider an NDA filing and potential review without having to complete a confirmatory study to form the basis of approval. Again, we are encouraged we have accomplished this goal by obtaining feedback from the FDA that confirms our Phase II data set is sufficient for them to consider an NDA filing.

Once an NDA submission is accepted by the FDA, it then begins the review process. It is in the FDA's purview to determine the type of review to apply based on the totality of the data included in an NDA. This means that the FDA can choose to review an application under the accelerated approval pathway, on the basis of a surrogate end point such as dystrophin, or under a full approval pathway, on the basis of a clinical end point such as the six-minute walk test. As part of our application, the FDA will have the totality of our data available to it including a robust biochemical effect in the form of dystrophin production, positive clinical outcome data including the six-minute walk test results, and a favorable safety profile in patients who will have more than two years of treatment at the time of submission, as this week represents the two-year mark of the first patients who were dosed in our Phase IIB study.

As part of our dialogue with the FDA, including two end of Phase II meetings, we asked for specific feedback on the acceptability of dystrophin as a surrogate end point to better understand whether the FDA would review our application under an accelerated approval pathway, as we were not sure if they would consider the acceptance of an NDA filing on the basis of our clinical outcomes data and felt that accepting dystrophin as a potential surrogate marker that would reasonably predict clinical benefit would be one possible pathway that they would consider. In response the FDA stated it would review the potential for dystrophin as a surrogate end point during the review following their acceptance of the NDA.

By considering the acceptance of an NDA for filing, it indicates that they would evaluate the totality of the data and consider all pathways for approval of eteplirsen and will determine if the agency's review will be under an accelerated

approval pathway or a full approval pathway after an NDA filing. During our recent meeting we had a productive discussion about dystrophin as an acceptable surrogate end point and the agency shared feedback on what it would like to see in the application including information on the methodology of our analyses and the potential verification of the dystrophin quantification data. While we have not yet received meeting minutes and we expect further discussion with the agency in the coming months, we believe based on the feedback so far we have received to date that we will be able to address their needs in our NDA submission.

Now that I've outlined the submission and review process specifically, I'd like to describe the key activities and communications that will take place over the next year to 18 months as it relates to eteplirsen. As we stated we plan on submitting a New Drug Application for eteplirsen in the first half of 2014. The specific timing of the NDA will be based on our continued interactions with the FDA and clarification of any additional information they may need as part of an NDA submission as it relates to our dystrophin data set and requirements for the CMC section of an NDA.

Approximately 60 days after the NDA submission, the agency will provide notice of whether or not they accept the application for filing. If the application is granted a priority review, the FDA will aim to make a decision on approval of eteplirsen within six months following an NDA filing as compared to a standard 10-month review.

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To sum up our progress to date we believe the FDA's willingness to consider an NDA based on our Phase II data set represents a tremendous achievement for our Company.

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ED KAYE, SVP & CHIEF MEDICAL OFFICER, SAREPTA THERAPEUTICS, INC.: Thanks, Chris.

We have very pleased to have received this feedback from the FDA and to know the FDA is open to considering an application for eteplirsen. It provides us the clarity we need to implement critical clinical and regulatory activities in the near term. We believe that we are well prepared for the tasks ahead and we appreciate the urgency to make this drug available to boys with DMD.

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Moving on to recent clinical activities, last quarter, we reported very encouraging 84-week results from the long term Phase IIB extension study with eteplirsen. We think the most important takeaways from these updated data are that we continue to see a stabilization of walking ability across all of the ambulatory eteplirsen-treated boys and the long term data offer increasing confidence in the potential

benefit and safety profile of the drug. As we know from the natural history in DMD, we would not expect to see stabilization in these boys who are now on average about 11 years of age.

Also as you know, we have reported individual patient results showing increases in novel dystrophin expression in all of the patients and also a stabilization of walking ability and those boys who remain evaluable on the six-minute walk test. We continue to follow the two boys who essentially lost ambulation by the time we were able to confirm dystrophin in their muscle biopsies. Data from other clinical outcome measures such as pulmonary function suggests that these boys are also stable.

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We are also making good progress with plans for the confirmatory study of eteplirsen and we continue to expect to initiate dosing in the first quarter of next year. We know that the six-minute walk test will be a primary end point in the study, and therefore we will be enrolling boys who are still remaining ambulatory.

We are continuing to have discussions with the FDA about other design elements including the use of a placebo or a matched control patients who are amenable to treatment with exon skipping therapies targeted to other exons such as exon 45, 53, 50, and 44. While we do not yet know for sure if the FDA will accept this masked arm as a potential control for the study we are planning to include this arm nonetheless. Early on it will help us collect additional natural history data and it may accelerate our development and approval path for our follow on exon skipping therapies.

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TIM LUGO: Thanks, and can you maybe just discuss well the nature of any questions in your mind that may be answered when you receive the meeting minutes coming up?

CHRIS GARABEDIAN: ***Well Brian, obviously, we felt comfortable with putting out a communication following the meeting and so if there was a lot of information that we were awaiting in the minutes, we probably wouldn't have been as confident in communicating the plans for a submission of an NDA.*** But there are always details to be worked out and as we prepare the activities in the sections of the NDA, there is a dialogue just clarifying exactly what they would like to see in that NDA. And we've indicated that there may be further information and specific information about the dystrophin methodology and the how it was quantified in verification of that. But again, as we stated, we believe all of the potential requests we could still meet and submit our NDA in the first half of next year. We don't know the details at this point. We will look for some of that in the meeting minutes and there will likely be ongoing discussion for us to clarify exactly what they're looking for in the NDA. But again, as we get more

information and we're more clear on what is going to be required we will communicate that.

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ROBIN KARNAUSKAS, ANALYST, DEUTSCHE BANK: Hi guys thanks for taking my question. I was just wondering on the accelerated approval versus full approval, how common is that for the FDA to give that option out after an FDA meeting when you file for accelerated approval for them to remind you that it could be full approval. And then maybe if you give us some precedence for an accelerated approval filing originally turning into a full approval. …

CHRIS GARABEDIAN: Robin so there's a few questions in there, so I think we can address all of them. ***So first regarding how common it is, again I'd say what's less common is a company going to the FDA and clarifying if a data set is sufficient for acceptance of an NDA submission. We believe that because it was a small Phase II study but because the results were quite robust, both biochemically, clinically and safety wise, that it might warrant a review and potential approval. And so this is why the Company has put a lot of effort into getting guidance from the FDA on the feasibility of this data set to form the basis of approval.***

Now we focused initially on dystrophin as a surrogate under an accelerated approval pathway because it was our primary end point. We had every patient that showed dystrophin. We believed we had levels that were high and kind of reproducible and consistent across many samples. ***And we believe we had, and still believe we have a very strong basis that the dystrophin that we're producing is validating the clinical outcomes that we're seeing and should be acceptable as a surrogate end point under the accelerated approval pathway***. So we focused on that and we believed that the FDA might say dystrophin could be considered as a surrogate end point but we're not going to weigh in on the clinical outcomes and the rest of the data set as a basis of approval.

What we ended up getting was a yes answer to the bigger question which is an NDA filing acceptability based on the totality of our data. So while they decided to say that they would make a decision on dystrophin after an NDA filing and during the review process, they told us yes, we would consider an NDA filing on the totality of the data and that would include the clinical outcomes. So again, many sponsors who might do two large well controlled studies where they know based on the guidelines that it would be acceptable for an NDA filing, ***we wanted to get guidance apriori before we put all of the effort that Ed described is needed to submit an NDA. And so we got our answer and this is why we're very confident in moving forward with that NDA submission.*** ... But I'll have Ed describe the various approvals that Genzyme, his experience at Genzyme demonstrated on relatively small samples in various forms.

ED KAYE: Sure. …I think one important aspect to remember is when they say they look at the totality of the data, if you can show a clinical improvement and the drug is acting in the same biologic way that you expect it such as you have a dystrophin deficiency, you replace it, you see a clinical benefit, then it helps again to allow them to make a decision. Because you have scientific and clinical evidence that the drug is doing what you're supposed to do.

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BILL TANNER: And then as it relates I don't know if you can, and I've not looked at your paper yet, I don't know if its mentioned in there, but can you comment as to the extent of a correlation between the dystrophin production and the six minute walk test and whether or not you think that that's going to be something that is actually going to be important to have?

CHRIS GARABEDIAN: Bill, this is Chris. Here is what we are ready to say at this point. Every patient showed dystrophin levels positive fibers between 30% to 60% after 48 weeks of treatment. Every patient whose evaluable on six minute walk has stabilized their six minute walk test distances. ***We think that the range we're showing on immunofluorescence of dystrophin is clearly above that threshold that Ed's describing because every patient that we follow, the individual patient data we've shared have all stabilized.*** The last 84 week update from the last time point before dystrophin was confirmed, not one patient declined by more than 5% from that last time point before dystrophin was confirmed at the levels I'm describing. ***So again, we think that correlation is very clear. We've shared all that data with the FDA. And again, we're confident that that helps validate this dystrophin end point***, but obviously, that's ultimately the FDA's call and they've indicated that that will be a review issue before they make that determination.

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JOSEPH SCHWARTZ, ANALYST, LEERINK SWANN & COMPANY: Hi, thanks very much. I was wondering about the two patients that lost ambulation and they are discussed in a little more detail in the Annals of Neurology paper, and in particular I was struck by the fact that they had a similar increase to the mean in terms of the dystrophin that was put back into their muscles. I would have thought that based on the discussion in there regarding MRI assessments et cetera that their muscles seem to be more fatty and fibrotic. So how is it that they are able to take up the dystrophin in the muscle and then still advance? What does that say about the limits of the technology?

ED KAYE: Yes, no, I think remember that the biopsies were done in the upper extremities and the boys lost ambulation. So we weren't testing, to finalize we weren't testing the upper extremity. So I think what happens is that the muscle that's remaining is able to take up the dystrophin and produce novel dystrophin, but unfortunately once that muscle is fibrotic, we can't repair it and based on all of

> the data it appears that it was too late for these boys. We couldn't get dystrophin. We couldn't change the process and turn the disease process around fast enough. But what we have seen is that the upper extremity function seems to have been stabilized and pulmonary and cardiac function is also stable, so the remaining muscle seems to be responding to the dystrophin, so I wouldn't look at this as they are non-responders. I think their legs were too far gone before we could make a difference.
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> OPERATOR: Thank you. I will now turn the call back to Chris Garabedian for closing comments.
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> CHRIS GARABEDIAN: Okay, thank you, operator. I want to reiterate that we are very pleased to have achieved some clarity with the FDA on the regulatory path forward for eteplirsen, and we are hopeful that our application will be filed for review and that eteplirsen will be considered for approval. …

(emphasis added).

22. On August 15, 2013, Sarepta presented at the Canaccord Genuity Growth Conference. Defendants Garabedian, Kaye and Mahatme participated in the call.

> CHRIS GARABEDIAN: Yes, sure, Ritu. So, obviously Sarepta as a company focused on RNA technology and our lead program is in Duchenne muscular dystrophy. We have a Phase II program that's ongoing of which we shared our latest data with the FDA and asked them if they would consider an NDA filing based on our Phase II data. And we had good, productive discussions with the FDA, and they indicated recently that we announced on July 24 that they are open to considering an NDA filing based on our current data set. So, we announced that we will be submitting our NDA for our drug eteplirsen for the treatment of Duchenne muscular dystrophy for those amenable to an exon 51 skipping drug. So that's the latest news we've shared.
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> RITU BARAL: Did you count the minimum number of fibers?
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> RITU CHRIS GARABEDIAN: We did. …
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> I think the bigger question or I guess the debate around dystrophyn is what's the magic number? What's the level that we can have confidence in that will translate to clinical outcome? And we believe we have that in our study. Even though it's 12 patients, every single patient in which we have confirmed dystrophyn from the last time point in which dystrophyn was confirmed, they've all been completely stable.

23. On September 9, 2013, Sarepta presented at the Morgan Stanley Healthcare Conference. Defendant Garabdian spoke.

> [Garabedian] So, Sarepta is an RNA-based technology company. Our lead program is in Duchenne muscular dystrophy. Our lead product in development, eteplirsen, is in a Phase IIb extension study. We recently shared that Phase II data with the FDA over the last six months and they recently in July communicated that they are open to considering an NDA filing based on the data we've shared with them or not requiring a confirmatory study before they would consider an NDA filing. So, we had communicated that we would be submitting an NDA, new drug application, in the first half of next year. We have an upcoming CMC meeting on the calendar with the FDA. And that's the update for now.
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> SARAH SUBCOMB: Okay, great. Are there any questions from the audience?
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> ***So last month or a few weeks ago, there was some data released from one of your competitors that caused a little concern. And I think the general concern was that it's actually a similar technology but benefits weren't seeming to correlate that well with dystrophin production. So, do you have a view on whether this is going to kind of hurt your chances at the FDA or the differences between your drug and Prosensa's drug and to why you're not seeing a great correlation there?***
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> CHRIS GARABEDIAN: We don't like to comment on competitor data sets, but we do look forward to the emerging data set that we expect in the coming months drive the person.
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> ***Look, we believe that our data set stands by itself in that we have shown not only a very favorable safety profile in patients who have taken the drug for nearly two years, we also have biochemical data that's very consistent across genotypes, across samples within patients and then across all 12 patients in our study in a range that has really been unprecedented of a 30% to 60% range of dystrophin positive fibers. And we're seeing clinical benefit conferred by that dystrophin production. So, we think it's very important to show all three of those elements -- a strong safety profile that allows for chronic dosing in a pediatric population, biochemical response that is consistent across 100% of the patients at levels that would be deemed meaningful, and a clinical benefit that correlates, that supports the dystrophin that we're seeing.***
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> I think, you know, we would like -- we did hear about the recent top-line dystrophin where upwards of more than 25% to 40% of patients did not show dystrophin increases by any measure that they were evaluated on.

So, the criteria was any dystrophin increase which was not defined by any measure of either RT-PCR immunofluorescence or Western blot. That's a very low threshold to show dystrophin and they still didn't meet it in 100% of the patients using that criteria. ***So, conversely, we were showing 100% of the patients at 12 weeks, even in our previous UK study, at lower doses by RT-PCR. So, again, we think we have a strong data set that's very robust and consistent across the population. And that's the message that we're going to go out to the market with.***

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SARAH SUBCOMB: Okay, great. And then just a quick question about the inclusion criteria. Are you changing your baseline six-minute walk inclusion criteria? And the reason I ask is, in the Phase II study with the two kids in the 30 milligram per kilogram arm that declined very rapidly despite the fact that they seem to have pretty good dystrophin production, so how are you trying to kind of eliminate that variable?

CHRIS GARABEDIAN: Yes, so we're not disclosing specific details on inclusion criteria but we have discussed the idea that the Phase II study may have had too relaxed of a criteria what we went down. We allowed patients anywhere from 200 meters to 400 meters walk. And a lot of the natural history that has emerged even in this past year after we designed the Phase II study really suggests that you should try to limit the lower end of that inclusion criteria to some say no less than 350, some say no less than 330. So we are still evaluating that because we also want to be able to enroll as many patients as is legitimate to help with enrollment times, etc. So we haven't determined the details, but we may be changing those inclusion criteria to have a more predictable rate of decline and to avoid maybe those that are too far gone like we found with the twins in our Phase IIb study.

However, we are looking at a larger cohort. We do expect balance in the arm. This is why, if we did enroll patients who are closer to the precipice of losing ambulation, a placebo arm becomes problematic because you start to be borderline unethical of enrolling a patient into a placebo arm that may be declining and have irreversible loss of ambulation. So, that's where, on an untreated cohort, where we don't have drug ready for exon 50, 53, 45 and 44, then that would be reasonable to have a balanced arm where, even if we lost some patients in the eteplirsen treated, you would expect to have a similar number in the untreated cohort.

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SARAH SUBCOMB: Maybe piggybacking off of that question a little bit, you've got a bunch of other exon targeting drugs in your pipeline and you've spoken about dystrophin as being an eventual surrogate for kind of a class approval for all these drugs. What gives you confidence that, with all these other exon skipping drugs, you're getting the same levels of functional dystrophin?

> CHRIS GARABEDIAN: Well, obviously the next real proof of concept in that is the dosing of those other exon skipping drugs in patients to prove that we can produce dystrophin, and ideally dystrophin levels that are in a comparable range of what we show with eteplirsen at a standard dose.
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> The reason we've been so vocal about the importance of dystrophin is that to think about streamlined development of follow-on exons and eventual class approval, it's based on the notion that that is the most reasonable pathway to gain approval in the most rapid manner of follow-on exons because everybody understands the limitations of rare genetic subtypes in a disease like Duchenne where it's infeasible and likely impossible to enroll enough patients to power a study for a clinical benefit like six-minute walk test. You get into confounding issues of do you start doing dose ranging and optimization around each exon target.
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> So, our premise is that the in vitro exon skipping efficiency can be done in a way to optimize sequences across all these are genetic subtypes. And if that can translate to a dystrophin at a standard dose in human muscle tissue, that we can then start to extrapolate what we've proven with let's say eteplirsen of the benefit of clinical outcomes that we're seeing.
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> Look, we know that, when you approve drugs on the basis of a surrogate like dystrophin, that the FDA will likely want sponsors to follow those patient postmarketing. We would intend to do that anyway for safety, for other clinical outcomes of stability, even beyond six-minute walks but probably pulmonary function and other measures. So we know that the answers will be there eventually, even in the rare genetic subtypes, but that it's not a reason to stop access to these drugs and commercial approval of these drugs because we can't enroll enough patients to find a clinical benefit.

(emphasis added).

24. On October 17, 2013, Sarepta presented at the DMD Development Program Update. Defendants Garabedian and Kaye spoke at the conference:

> [Garabedian:] ***Before I turn the call over to Ed Kaye to provide the latest clinical data with eteplirsen and our plans for our confirmatory study, I'd like to highlight the strong data set related to our technology and the proof of concept that we are producing meaningful levels of dystrophin, and that the doses we are studying in the clinic are producing the desired effect in support of our drug's mechanism of action and our ability to produce the essential protein dystrophin that is lacking in patients with Duchenne and the root cause of the disease.***
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CHRIS GARABEDIAN: ***Thanks, Ed. And before I move on to describing our Let's Skip Ahead website, I wanted to address a question that has come up a lot over the last few weeks as it relates to the failed drisapersen studies and what it means to our program.***

While the failed studies with drisapersen were a disappointment to the DMD community, we believe it underscores how important it is to have a chemistry that does not have dose-related toxicity that may prohibit a dose that is active enough to produce a clinical effect.

Eteplirsen uses a very different chemical structure as what we call the drug's backbone, and this unique chemistry allows us to achieve doses in our studies that are five- to eightfold greater than those doses studied in the drisapersen trials. And the dystrophin data set I described earlier underscores the importance of achieving a dose that produces the type of robust and consistent response that we have seen in our studies.

We understand the disappointment of the drisapersen trial participants, particularly those who were discontinued from drisapersen treatment upon the announcement of the failed clinical trials. While we understand this disappointment, we are exploring the feasibility of addressing these patients who were in the drisapersen trials.

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So in summary, we have a lot going on for the remainder of this year, and for the next six to nine months, as we have several FDA meetings. We are currently putting together sections of our NDA and planning for an NDA submission in the first half of 2014, and clarifying exactly what needs to go into that NDA with the FDA in these subsequent meetings.

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JANE LARKINDALE, VP RESEARCH, MUSCULAR DYSTROPHY ASSOCIATION: …

We have a number of questions related to the GSK data announcement. I was wondering if you would be able to address some of the repercussions from that data announcement. How do you think the failure of that large trial will impact your chances of getting accelerated approval, and do you still think the six-minute walk test is going to be the best endpoint for your larger studies?

CHRIS GARABEDIAN: So Ed, why don't you address the six-minute walk test vis-a-vis the drisapersen failed studies, and then I can address the accelerated approval question.

ED KAYE: Sure. Jane, I think, obviously we really can't comment on the drisapersen study because we were not participants and don't really understand all the aspects of that.

But I think it has caused concern about whether the six-minute walk test is an appropriate clinical test for DMD boys. And I think the short answer is we still firmly believe that it is a good test. In our review of all the data and certainly the data in our hands, we found it to be the most sensitive end point.

I think an important aspect that we focused on is really the patient selection to use this endpoint, because eteplirsen is really focused on maintaining the six-minute walk test distance. We are not improving on the six-minute walk test, but we are trying to keep the boys stable and preventing further deterioration.

So it's really important to have a cohort that you would expect to deteriorate during the time of the study. And so, we focused on the over seven, and there has been a number of Natural History Studies just this year alone that have confirmed that boys over seven years of age really do continue to deteriorate, on average, from 40 to 80 meters. And even in the recent drisapersen study, the boys who were over seven years of age deteriorated, on average, between 75 and 82 meters.

So I think by having a very homogeneous population that should decline during the focus that we can demonstrate that these boys are stable, then that, I think, should be a good study design. And Chris, I will let you comment further.

CHRIS GARABEDIAN: Great. ***Yes, so Jane, on your question related to accelerated approval, we believe these two drugs are very different, so in terms of will the FDA look differently upon our NDA submission in light of the drisapersen data, again, we think our data set is very differentiated, as I described earlier with our dystrophin analysis. That is because they are very different chemical entities.***

Our drug chemistry backbone is very different, and we think the data will stand alone and stand on its own in front of the FDA and won't confuse our clinical data set because, again, we had a dose that was five- to eightfold higher with a different drug chemistry, so we do not think that the failed studies of drisapersen are an indictment at all on exon skipping or our drug's ability to see that exon skipping, as we demonstrated in 100% of our patients.

So with that, again, our plans are intact to submit an NDA, and we will have -- obviously continue to follow these 12 patients. Still plan to enroll our confirmatory study, and of course, we are disappointed that a potential treatment has been hampered and may affect the timing or even the possibility of pursuit for an approval of that drug. And so, we think there is even more urgency on Sarepta's part to do everything possible to make eteplirsen available as soon as possible through whatever mechanism the FDA allows through the NDA process.

(emphasis added).

25. On October 30, 2013, the price of Sarepta's stock fell $3.26 to close at $38.92, a 7.8% decline from its closing price of $41.89 per share on October 29, 2013, following reports published that day questioning the significance of the data set from Sarepta's Phase IIb study of eteplirsen.

26. For instance, on October 30, 2013, the investment website, *Seeking Alpha* published an article entitled "Short Sarepta: DMD Prognosis is Dismal." The article stated in part:

> Investors' bullish outlook on eteplirsen went into hyper-drive last month when **GlaxoSmithKline** (GSK) and **Prosensa** (RNA) announced that their DMD candidate drisapersen failed to meet its primary endpoint in a pivotal Phase III trial. Despite showing promising Phase II results and increased dystrophin expression as well, drisapersen wasn't even close to outperforming placebo in terms of improving walking performance in a larger sample of DMD patients. This news sent Sarepta shares into orbit (up 25% in one day) because it essentially gives the entire DMD market to eteplirsen, pending approval.
>
> **So everything is good, right? Eteplirsen will be approved, etc…**
>
> While Sarepta is publicly spinning a compelling tale for investing in eteplirsen's prospects, the science tells a wholly different story. In fact, I am confident eteplirsen is not going to receive early approval by the FDA, and will in fact go on to fail in a Phase III trial.
>
> The worst part is that the company seems to share my bearish sentiment, which is why they are pushing for early approval based on an extremely small Phase II trial.
>
> To understand what's really going on, we need to look past the company propaganda. By contrast, the truth can be found in the dense, off-putting, boring scientific literature. Indeed, this is probably why the market has failed to recognize eteplirsen's imminent doom, and instead has pushed the stock to new heights.
>
> With that in mind, I'll do my best to distill the important, and wholly misunderstood, aspects of eteplirsen. And subsequently, why the drug is actually in deep trouble and Sarepta knows it.
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> ***

Didn't eteplirsen significantly improve walking performance in clinical trials?

The simple answer is no. Despite multiple reports that eteplirsen did in fact show a clinically significant improvement in the 6mwt in a Phase II trial, the opposite is true.

Why did investors believe eteplirsen improved walking performance?

Again, the answer is simple. People didn't understand the statistics, and the company didn't exactly promote results that ran counter to their goal of selling stock.

The problem arises largely because the Phase II trial conducted by Sarepta only included 12 patients. Such a small sample size is problematic for a number of reasons. Chief among these is the fact that small sample sizes lead to low statistical power by default, and tend to have weird, non-normal distributions. And Sarepta's study was no different.

In their analyses including all 12 patients, eteplirsen **failed** to show a significant improvement in the 6mwt when the data was properly analyzed. Going directly to the actual peer-reviewed paper, it reports for the 6mwt at 24 weeks:

*"As data distribution analysis revealed severe violation of normality assumption, the analysis was repeated using ANCOVA for ranked data, showing **no** significant differences between the 2 eteplirsen and placebo cohorts."*

At 48 weeks, the paper states the following:

"As data distribution analysis revealed severe violation of normality assumption, the analysis was repeated using ANCOVA for ranked data, showing no significant differences between the 30 mg/kg and placebo cohorts but a statistically significant difference between the 50 mg/kg and placebo/delayed cohorts ($p < 0.016$)."

So it's a dosing and time issue, right? Eteplirsen works at the higher dose, correct?

While that would be a nice result, it's not quite right. The truth is that two boys in the lower dose group (30mg/kg) severely skewed the group's distribution. Sarepta researchers thus argued that the two boys should be excluded from further analyses because they are "outliers" due to their age, height, and rapid disease progression.

Regarding the minimally significant result in the higher dose, you should keep in mind that this result is not corrected for Type I error, which is explicitly stated in the paper. In other words, this "significant" result for the higher dose could very well be what's known as a false positive, and is probably not significant after

applying Type I correction. No way to know with such a small sample size. But again, Sarepta hasn't gone out of their way to inform the general investor of such statistical issues...

What happens when the boys are excluded?

At 48 weeks, the 6mwt suddenly becomes highly significant ($p < 0.0001$) for the low dosage cohort compared to placebo.

So are the two boys really outliers? Which analysis is correct?

First off, I need to explain what an outlier means in statistical terms. An outlier is generally a datum that lies well outside the distribution of the overall dataset. Legitimate reasons to deem a datum an outlier include, incorrect data entry, suspicion of incorrect measurement, methodological problems, and so on. There are ways to specifically test for outliers and statisticians do tend to get grumpy when you don't have a good reason to include a particular datum in your analysis.

Why? Because excluding data that doesn't fit your hypothesis is tantamount to cherry picking. And unfortunately, that's what Sarepta did with their Phase II data.

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The twin boys showed dramatic increases in dystrophin production

The entire hypothesis is that eteplirsen increases dystrophin production, which in turn, should increase ambulatory performance. Yet, these two boys showed marked increases in dystrophin-positive fibers at both weeks 24 (20.8% and 15.9%) and 48 (48.3% and 43.6%).

The significant increase in dystrophin production thus flies in the face of the researchers' decision to exclude them as "outliers" in the 6mwt. In fact, I would be much more bullish on eteplirsen's prospects if these extreme DMD patients showed stable ambulatory performance when taking eteplirsen, rather than getting worse. Biologically, these two boys were not outliers, and should never have been excluded from the analysis. In all fairness, the peer-reviewed paper did report the correct analysis, but the company has actively chosen to promote the more liberal interpretation of the results.

So let me get this straight. Dystrophin production increases yet ambulatory performance worsens?

Yes. This is why Sarepta is pushing the FDA to accept dystrophin production as their primary endpoint in an NDA filing, not the 6mwt that drisapersen was evaluated on. The FDA has yet to agree this is an appropriate primary endpoint, and I doubt they ever will.

Bottom line: Eteplirsen does not appear to provide a clinically meaningful benefit in terms of increased walking performance when the data is analyzed properly. To get the desired result, Sarepta had to inappropriately massage this miniscule dataset, and forgo any correction for Type I error.

But that's not all…

Why Sarepta chose such a small sample size is beyond me. Phase II clinical trials for drisapersen included 48 subjects and didn't involve any unwarranted data massaging. Sarepta's design, by contrast, is so small that Type I error corrections cannot be applied without losing a big chunk of statistical power, which is probably why it wasn't performed. The FDA tends to like to err on the side of caution, and it's common for them to ask for the more conservative p-values post-correction. My bet is that Sarepta's story falls apart at that point.

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Is Sarepta aware of these problems?

Sarepta is most definitely aware of these issues. They did report the correct analyses in their peer-reviewed work, and were upfront about not correcting for Type I error. It just wasn't trumpeted in their marketing materials to investors.

27. Then, on November 12, 2013, Sarepta issued a press release announcing an update on developments concerning eteplirsen and its intentions to submit an NDA:

Sarepta Therapeutics Announces FDA Considers NDA Filing for Eteplirsen Premature in Light of Recent Competitive Drug Failure and Recent DMD Natural History Data

FDA questions dystrophin as a biomarker due to failed studies of other investigational drugs for DMD;

FDA questions 6-minute walk test results for eteplirsen, suggesting study population should be stable over two-year timeframe due to recent natural history data;

FDA requests further discussion on endpoints, design of confirmatory clinical study

CAMBRIDGE, Mass. – November 12, 2013 – Sarepta Therapeutics, Inc. (NASDAQ: SRPT) today provided an update on its discussions with the U.S. Food and Drug Administration (FDA) regarding its planned New Drug Application (NDA) submission and confirmatory clinical study with eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). Citing recent developments since Sarepta's last meeting with the agency, including a failed study with a competitive product and recent natural history data in DMD, the

FDA indicated the new data raise "considerable doubt" about both the dystrophin biomarker and the supportive clinical efficacy assessed on the 6-minute walk test (6MWT) in the Phase IIb clinical study of eteplirsen. As a result of these recent data, the FDA stated that they "currently consider an NDA filing for eteplirsen as premature."

"We are very disappointed with the FDA's decision to reconsider their openness to a potential NDA filing based on our current data and the resultant impact this change may have on our efforts to achieve an earlier approval of eteplirsen," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We strongly believe in the potential of eteplirsen to address a serious unmet medical need in DMD and we are committed to its development. …

The FDA provided the feedback in pre-meeting comments and clarified them in a meeting with Sarepta that took place late last week to discuss the eteplirsen clinical program.

Excerpts from the FDA's pre-meeting comments on reconsidering an NDA filing included:

"Since our last meeting, a large phase 3 trial of drisapersen, a drug with a similar mechanism of action, was reported to be negative, despite increased expression of dystrophin. The disconnect between increased expression of dystrophin and clinical efficacy for drisapersen, combined with previous negative reports for PTC124, another drug thought to act by increasing dystrophin, raises considerable doubt about the biomarker, and consequentially, its ability to reasonably likely predict clinical benefit."

"…the quantity of dystrophin that might be necessary to be considered reasonably likely to predict clinical benefit is even less clear; small or perhaps even moderate increases are seemingly not enough, at least in the subpopulation of boys studied so far. An adequately validated quantitative assay for dystrophin now seems a prerequisite to further consideration of the biomarker as supportive of approval. Since our last meeting, our concern about the shortcomings of your current quantification methods has grown."

"Recent natural history data in DMD indicate that a baseline 6-Minute Walk Test (6MWT) □350 meters predicts continued general stability for such patients, not the 75- to 83-meter yearly decline you suggest in the meeting package. Thus, considerable doubt is also cast on the efficacy support provided by your ongoing open-label study (4658-us-202, 96-week data submitted), in which baseline 6MWT was >350 m for all patients."

"…the expected variability of 6MWT values appears sufficient to explain differences between arms on which the post-hoc analysis was based. Because of this, together with our lack of confidence in the capacity of your dystrophin

biomarker to predict clinical benefit, we currently consider an NDA filing for eteplirsen as premature."

Additional excerpts from the FDA's pre-meeting comments on the eteplirsen confirmatory study design included:

"Recent trial failures in DMD suggest it may be productive to re-examine study enrollment criteria and endpoints."

"...it seems worthwhile to consider selection of other endpoints and/or populations for the next trial of eteplirsen. We stress that we would still accept 6MWT in an appropriately powered study; however, because 6MWT excludes both younger boys who cannot perform such a demanding test, and older boys who are no longer ambulatory, we are concerned that seemingly avoidable limitations on enrollment could undermine study feasibility. Many possible combinations of endpoints and subpopulations appear possible. Motor scales that measure a broader range of function and demand less sustained effort than 6MWT could be appropriate for a much wider range of boys, perhaps including non-ambulatory boys. To allow inclusion of a broader range of patients, a study could also be designed that mathematically combined findings from, for example, an ambulation endpoint in less advanced patients with findings from an upper-limb or respiratory endpoint in more advanced patients. We remain open to consideration of endpoints and populations you may suggest."

"...we believe that a placebo-controlled trial would be the most likely method for developing interpretable evidence of efficacy for eteplirsen, because efficacy endpoints in DMD are effort-dependent and susceptible to bias, and the natural history is highly variable and has recently improved with steroid use and advances in ancillary care. We would like to discuss the perceived barriers to conducting such a trial with you."

The FDA's request to discuss different clinical endpoints, combined endpoints, and different DMD subpopulations for a confirmatory clinical study, along with their questions about dystrophin as a biomarker and the need for a placebo-controlled study, will delay the initiation of dosing in the eteplirsen confirmatory study until at least the second quarter of 2014. A follow up meeting with FDA has been scheduled to take place this month to discuss the confirmatory study design.

28. That same date, November 12, 2013, the Company held its earnings conference call for the third quarter ended September 30, 2013.

[Garabedian:] Last Friday, at 4 PM Eastern Time, we had a meeting with the FDA that was intended to focus on finalizing our confirmatory study design with eteplirsen. We were very pleased with the FDA's involvement at this meeting, in that they extended our allotted meeting time from 60 to 90 minutes, and there were about 25 participants from the FDA, including Janet Woodcock, the Director

of CDER; John Jenkins, the Director of Office of New Drug; Bob Temple, the Deputy Director of Clinical Science for CDER; and Ellis Unger, the Director of Drug Evaluation I; along with Eric Hastings and Ron Farkas, the acting Director and the Clinical team leader for the Division of Neurology Products. So we were very thankful to have the FDA's active involvement in this program on the eve of a holiday weekend, and believe it highlights the importance of this program within the Agency. While the express purpose of the meeting was to finalize our confirmatory trial design, the Agency provided unsolicited comments related to a change in their thinking on the regulatory status of eteplirsen, our clinical data, and our clinical development plans, as a result of new data that they have considered and interpreted since our last meeting in July. Specifically, they indicated that since the last meeting with Sarepta, and based on the recent failed Phase III trial of drisapersen GSK and Prosensa's exon-skipping drug candidate for DMD, as well as citing negative reports for PTC's therapeutic drug, ataluren, another drug using a different mechanism of action to attempt to produce dystrophin, along with recently published natural history data on DMD that their concern has increased. And it has raised considerable doubts about dystrophin as a biomarker, and about our supportive 6-minute walk efficacy data.

As a result, they have shifted their stance, from being open to considering an NDA for filing, to now considering an NDA filing for eteplirsen as premature. We do not fully understand nor do we agree with many of the FDA's comments and new perspectives, as we believe our dystrophin data has been well-characterized and compares favorably to, and is differentiated to other dystrophin-producing technologies including drisapersen and ataluren. And our 96 week 6-minute walk test data is not typical of what has been characterized in the broader natural history literature, and in clinical studies including the placebo and failed drisapersen ARMs of their Phase III study. With regard to our 6-minute walk test efficacy data, the FDA's decision to reconsider being open to NDA filing since our last meeting is based in part on recent national history data that has led them to believe that DMD boys that can walk over 350 meters at baseline of a study in their words quote, predicts general stability for such patients, end quote. Not the 75 to 83 meters that we had described for them based on historical data, and what has been reported in the greater than seven-year-old population of the Prosensa and GSK case study across both the drisapersen and placebo arms, respectively. That study also had a population that represented a similar exon-51 amenable population in our studies.

They stated that as a result of the new natural history data that quote, considerable doubt is also cast on the efficacy supports, end quote of eteplirsen, which was provided to them through 96 weeks from our Phase II extension study, and that quote, it may be productive to re-examine study enrollment criteria and endpoints, end quote in connection with a start of a confirmatory clinical study. These new perspectives and their request to re-examine study enrollment criteria, endpoints, and subpopulations will result in a delay in our clinical program, and the earliest we would expect to begin dosing in a confirmatory study will now be the second quarter of next year. Additionally, the agency has reiterated their demand for a

placebo-controlled study because in their view quote, efficacy endpoints in DMD are effort-dependent and susceptible to bias, and the natural history is highly variable and has recently improved with steroid use and advances in ancillary care, end quote. Since the placebo-controlled trial would require a doubling of the sample size of our planned confirmatory study, and could pose problems obtaining IRB approval to obtain two surgical biopsies with general anesthesia in placebo patients in order to protect the blinding, this would significantly delay our ability to recruit an ambulatory age-appropriate exon-51 amenable population in the US. Recognizing the potential challenges of recruitment, the FDA suggested exploring additional subpopulations and endpoints, and were open to discussing novel approaches to designing a placebo-controlled study, although the type and extent of these endpoints and subpopulations remains open.

… To be clear, we will be pursuing a more traditional path of approval, while we concurrently try to persuade the FDA to reconsider the potential of an early filing strategy for eteplirsen. However, we know that many patients had put a lot of hope into our ability to convince the FDA that the drug deserves early approval, and had expectations that with the potential NDA filing in the first half 2014, that the possibility of an eteplirsen FDA approval could have come in late 2014 or early 2015. The delay of our clinical study and the request by the FDA to revisit enrollment criteria and endpoints, and to consider the possibility of subpopulations and combinations of endpoints pushes the potential timeline of an eteplirsen approval out two years or more.

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BRIAN SKORNEY, ANALYST, ROBERT W. BAIRD & COMPANY, INC.: Hi, good morning. Thanks for taking the question, and sorry to hear about the disappointing notification from the FDA. I guess, Chris, it sounds like a lot of what you are saying, you are still thinking about a potential for an early filing. Did the FDA leave any opening to reconsider this decision on the acceptability of an early filing? Or give us some color around what you think the path forward is here, and how hard and fast, the FDA is about say no to an early NDA?

CHRIS GARABEDIAN: …

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… So we will continue this compiling those documents and NDA modules, in the hopes that we can convince them over the next weeks to months to reconsider. So I think, we believe the door is opened for them to reconsider this. But at this time what we have disclosed is all we can provide us an update at moment. But we do believe, ***just like the FDA kind of changed their position from July to now***, we think, as we are able to describe more of the data, that they may change their position yet again.

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> KIMBERLY LEE, ANALYST, JANNEY MONTGOMERY SCOTT: Good morning, thanks for taking the questions. Apologize if I missed this, but what are your plans for the rest of the drugs in the pipeline? And what are the implications you think of, the results from the FDA on, with regards to the rest of your pipeline and clinical endpoints and study designs? Thank you.
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> CHRIS GARABEDIAN: Yes, Kim, that is -- it's a great question, because ***our entire program was largely predicated on dystrophin as an acceptable surrogate biomarker. And so, we were going to do that with the eteplirsen study, because we can enroll enough patients to show that clinical benefit correlated to the dystrophin that we are capturing. Again, with the FDA calling into question dystrophin as a biomarker, we don't believe that is something that can be a firm decision. Because it would render us unable to get other drug approved for the rare exon, because we would not be able to power studies on a 6-minute walk or these other endpoints.*** I just described how difficult it is to enroll patients with eteplirsen amenable exon deletions. So this is why we need to work with the FDA closely, to come to an agreement and assessment of how to capture dystrophin in a way that they believe is validated. So that we can have streamlined approvals of the other exons. So it is difficult to say, where I sit today of what those development paths look like for the follow-on exons, and how the FDA will approach the potential regulatory approval of those other exons.

(emphasis added).

29. On this negative news, Sarepta's stock price fell $23.40 to close at $13.16 per share on November 12, 2013 on extraordinary volume, a decline of 64% from its closing price of $36.56 per share on November 11, 2013,.

30. An article in *Forbes* dated November 12, 2013, entitled "What The Delay of a Promising Muscular Dystrophy Drug Means for Patients, Investors and All of Biotech," commented on Sarepta's negative results:

> Unfortunately, great results from small trials have a history of not bearing out in larger studies. Even for rare disease drugs, this study was tiny. Worse, the Sarepta results only look good when two of the 12 patients are excluded – two boys were too sick to be helped by the drug. The FDA usually insists that clinical trials be presented in what is known as an "intent-to-treat" analysis, which means that if you even thought about treating a patient they need to be included when you do the math on the study's results. This is intended to keep scientists from lying to themselves, convincing themselves that a drug works when it doesn't. One biotech executive with a great deal of experience in rare diseases told me recently that this issue meant the data "would never fly" with the FDA. The recent failure

> of a similar, but less effective, drug from Prosensa and GlaxoSmithKline GSK -0.38% made the odds dimmer.
>
> What made FDA approval seem plausible is that the agency has just recently, as a result of a new law, gained the ability to use the "accelerated approval," which means the FDA can approval a drug conditionally, and then automatically yank it if it turns out the medicine doesn't work in larger studies. FDA could have approved eteplirsen, the thinking went, and then design some kind of study that allowed it to confirm the drug's efficacy – perhaps, Sarepta was suggesting, comparing boys on eteplirsen to those whose Duchenne was caused by a different mutation, preventing any boy who could benefit from having to get placebo.
>
> That's not going to happen now. The FDA wants a placebo controlled study. The good news is that for a small study, these results are still compelling, and if Sarepta does run a larger study there is a reasonable chance it will turn out positive. So after the stock has gotten beaten up over this, Sarepta could be a buy.
>
> Still, Sarepta Chief Executive Chris Garabedian should be chastened. He has been overly effusive and optimistic in his public statements about eteplirsen and the odds of an early approval to patient groups and investors. It's time for realism now, and he should make sure he has someone on his team who thinks that the odds of getting eteplirsen to market could be long and hard and full of challenges, so that he can make sure he does the right study. If eterplirsen is less effective than it seems – but still effective – the study designs he was discussing might not show it. A placebo-controlled trial will.

PLAINTIFF'S CLASS ACTION ALLEGATIONS

31. Plaintiff brings this action as a class action pursuant to Federal Rule of Civil Procedure 23(a) and (b)(3) on behalf of a Class, consisting of all those who purchased or otherwise acquired Sarepta securities during the Class Period (the "Class") and were damaged thereby. Excluded from the Class are Defendants herein, the officers and directors of the Company at all relevant times, members of their immediate families and their legal representatives, heirs, successors or assigns and any entity in which Defendants have or had a controlling interest.

32. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, Sarepta securities were actively traded on the NasdaqGS. While the exact number of Class members is unknown to Plaintiff at this time and

can be ascertained only through appropriate discovery, Plaintiff believes that there are hundreds or thousands of members of the proposed Class. Record owners and other members of the Class may be identified from records maintained by Sarepta or its transfer agent and may be notified of the pendency of this action by mail, using the form of notice similar to that customarily used in securities class actions.

33. Plaintiff's claims are typical of the claims of the members of the Class as all members of the Class are similarly affected by Defendants' wrongful conduct in violation of federal securities law that is complained of herein.

34. Plaintiff will fairly and adequately protect the interests of the members of the Class and has retained counsel competent and experienced in class and securities litigation. Plaintiff has no interests antagonistic to or in conflict with those of the Class.

35. Common questions of law and fact exist as to all members of the Class and predominate over any questions solely affecting individual members of the Class. Among the questions of law and fact common to the Class are:

- whether the federal securities laws were violated by Defendants' acts as alleged herein;
- whether statements made by Defendants to the investing public during the Class Period misrepresented material facts about the business, operations and management of Sarepta;
- whether the Individual Defendants caused Sarepta to make false and misleading statements during the Class Period;
- whether Defendants acted knowingly or recklessly in making false and misleading statements;
- whether the prices of Sarepta securities during the Class Period were artificially inflated because of the Defendants' conduct complained of herein; and
- whether the members of the Class have sustained damages and, if so, what is the proper measure of damages.

36. A class action is superior to all other available methods for the fair and efficient adjudication of this controversy since joinder of all members is impracticable. Furthermore, as the damages suffered by individual Class members may be relatively small, the expense and burden of individual litigation make it impossible for members of the Class to individually redress the wrongs done to them. There will be no difficulty in the management of this action as a class action.

37. Plaintiff will rely, in part, upon the presumption of reliance established by the fraud-on-the-market doctrine in that:

- Defendants made public misrepresentations or failed to disclose material facts during the Class Period;
- the omissions and misrepresentations were material;
- Sarepta securities are traded in an efficient market;
- the Company's shares were liquid and traded with moderate to heavy volume during the Class Period;
- the Company traded on the NasdaqGS and was covered by multiple analysts;
- unexpected material news about Sarepta was rapidly reflected and incorporated into Sarepta's stock price during the Class Period;
- as a result of the foregoing, Sarepta's common stock promptly digested current information about Sarepta from all publicly available sources and reflected such information in Sarepta's stock price; and
- Plaintiff and members of the Class purchased and/or sold Sarepta securities between the time the Defendants failed to disclose or misrepresented material facts and the time the true facts were disclosed, without knowledge of the omitted or misrepresented facts.

38. Based upon the foregoing, Plaintiff and the members of the Class are entitled to a presumption of reliance upon the integrity of the market.

COUNT I

(Against Defendants For Violations of Section 10(b) of the Exchange Act and SEC Rule 10b-5 Promulgated Thereunder)

39. Plaintiff repeats and realleges each and every allegation contained above as if fully set forth herein.

40. This Count is asserted against Defendants and is based upon Section 10(b) of the Exchange Act, 15 U.S.C. § 78j(b), and Rule 10b-5 promulgated thereunder by the SEC.

41. During the Class Period, Defendants engaged in a plan, scheme, conspiracy and course of conduct, pursuant to which they knowingly or recklessly engaged in acts, transactions, practices and courses of business which operated as a fraud and deceit upon Plaintiff and the other members of the Class; made various untrue statements of material facts and omitted to state material facts necessary in order to make statements made, in light of the circumstances under which they were made, not misleading; and employed devices, schemes and artifices to defraud in connection with the purchase and sale of securities. Such scheme was intended to, and, throughout the Class Period, did: (i) deceive the investing public, including Plaintiff and other Class members, as alleged herein; (ii) artificially inflate and maintain the market price of Sarepta securities; and (iii) cause Plaintiff and other members of the Class to purchase Sarepta securities and options at artificially inflated prices. In furtherance of this unlawful scheme, plan and course of conduct, Defendants, and each of them, took the actions set forth herein.

42. Pursuant to the above plan, scheme, conspiracy and course of conduct, each of the Defendants participated directly or indirectly in the preparation and/or issuance of the SEC filings, press releases and other statements and documents described above, including statements made to securities analysts and the media that were designed to influence the market for Sarepta securities. Such reports, filings, releases and statements were materially false and misleading in

that they failed to disclose material adverse information and misrepresented the truth about Sarepta's finances and business prospects.

43. By virtue of their positions at Sarepta, Defendants had actual knowledge of the materially false and misleading statements and material omissions alleged herein and intended thereby to deceive Plaintiff and the other members of the Class, or, in the alternative, Defendants acted with reckless disregard for the truth in that they failed or refused to ascertain and disclose such facts as would reveal the materially false and misleading nature of the statements made, although such facts were readily available to Defendants. Said acts and omissions of Defendants were committed willfully or with reckless disregard for the truth. In addition, each defendant knew or recklessly disregarded that material facts were being misrepresented or omitted as described above.

44. Information showing that Defendants acted knowingly or with reckless disregard for the truth is peculiarly within Defendants' knowledge and control. As the senior executives and/or directors of Sarepta, the Individual Defendants had knowledge of the details of Sarepta's internal affairs.

45. The Individual Defendants are liable both directly and indirectly for the wrongs complained of herein. Because of their positions of control and authority, the Individual Defendants were able to and did, directly or indirectly, control the content of the statements of Sarepta. As officers and/or directors of a publicly-held company, the Individual Defendants had a duty to disseminate timely, accurate, and truthful information with respect to Sarepta's businesses, operations, future financial condition and future prospects. As a result of the dissemination of the aforementioned false and misleading reports, releases and public statements, the market price of Sarepta securities was artificially inflated throughout the Class Period. In

ignorance of the adverse facts concerning Sarepta's business and financial condition which were concealed by Defendants, Plaintiff and the other members of the Class purchased Sarepta securities at artificially inflated prices and relied upon the price of the securities, the integrity of the market for the securities and/or upon statements disseminated by Defendants, and was damaged thereby.

46. Had Plaintiff and the other members of the Class known the truth, they would not have purchased such securities, or would not have purchased them at the inflated prices that were paid. At the time of the purchases by Plaintiff and the Class, the true value of Sarepta securities was substantially lower than the prices paid by Plaintiff and the other members of the Class. The market price of Sarepta securities declined sharply upon public disclosure of the facts alleged herein to the injury of Plaintiff and Class members.

47. By reason of the conduct alleged herein, Defendants knowingly or recklessly, directly or indirectly, have violated Section 10(b) of the Exchange Act and SEC Rule 10b-5.

48. As a direct and proximate result of Defendants' wrongful conduct, Plaintiff and the other members of the Class suffered damages in connection with their respective purchases and sales of the Company's securities during the Class Period, upon the disclosure that the Company had been making false and misleading statements to the investing public.

COUNT II

(Violations of Section 20(a) of the Exchange Act Against The Individual Defendants)

49. Plaintiff repeats and realleges each and every allegation contained in the foregoing paragraphs as if fully set forth herein.

50. During the Class Period, the Individual Defendants participated in the operation and management of Sarepta, and conducted and participated, directly and indirectly, in the

conduct of Sarepta's business affairs. Because of their senior positions, they knew the adverse non-public information about Sarepta's misstatements and omissions of material fact.

51. As officers and/or directors of a publicly owned company, the Individual Defendants had a duty to disseminate accurate and truthful information with respect to Sarepta's business, operations, financial condition and results of operations, and to correct promptly any public statements issued by Sarepta which had become materially false or misleading.

52. Because of their positions of control and authority as senior officers, the Individual Defendants were able to, and did, control the contents of the various reports, press releases and public filings which Sarepta disseminated in the marketplace during the Class Period concerning Sarepta's business, operations and results of operations. Throughout the Class Period, the Individual Defendants exercised their power and authority to cause Sarepta to engage in the wrongful acts complained of herein. The Individual Defendants therefore, were "controlling persons" of Sarepta within the meaning of Section 20(a) of the Exchange Act. In this capacity, they participated in the unlawful conduct alleged which artificially inflated the market price of Sarepta securities.

53. Each of the Individual Defendants, therefore, acted as a controlling person of Sarepta. By reason of their senior management positions and/or being directors of Sarepta, each of the Individual Defendants had the power to direct the actions of, and exercised the same to cause, Sarepta to engage in the unlawful acts and conduct complained of herein. Each of the Individual Defendants exercised control over the general operations of Sarepta and possessed the power to control the specific activities which comprise the primary violations about which Plaintiff and the other members of the Class complain.

54. By reason of the above conduct, the Individual Defendants are liable pursuant to Section 20(a) of the Exchange Act for the violations committed by Sarepta.

PRAYER FOR RELIEF

WHEREFORE, Plaintiff demands judgment against Defendants as follows:

A. Determining that the instant action may be maintained as a class action under Rule 23 of the Federal Rules of Civil Procedure, and certifying Plaintiff as the Class representative;

B. Requiring Defendants to pay damages sustained by Plaintiff and the Class by reason of the acts and transactions alleged herein;

C. Awarding Plaintiff and the other members of the Class prejudgment and post-judgment interest, as well as their reasonable attorneys' fees, expert fees and other costs; and

D. Awarding such other and further relief as this Court may deem just and proper.

DEMAND FOR TRIAL BY JURY

Plaintiff hereby demands a trial by jury.

Dated: January 27, 2014

/s/ Alan L. Kovacs

Alan L. Kovacs (BBO #278240)
Law Office of Alan L. Kovacs
257 Dedham Street
Newton, MA 02461
Telephone: (617) 964-1177
Facsimile: (617) 332-1223
alankovacs@yahoo.com

William B. Federman
FEDERMAN & SHERWOOD
10205 N. Pennsylvania Avenue
Oklahoma City, OK 73120
Telephone: (405) 235-1560
Facsimile: (405) 239-2112
wbf@federmanlaw.com

Attorneys for Plaintiff